kalydeco®

(ivacaftor) oral granules

5.8 mg • 13.4 mg • 25 mg • 50 mg • 75 mg

Treating the underlying cause. Another first worth remembering.

Talk to your healthcare provider to see if KALYDECO® is right for your loved one.

A brochure for parents and caregivers of children age 1 month to less than 2 years old

Use of KALYDECO in children aged 1 month to less than 6 months born from a pregnancy lasting (gestational age) less than 37 weeks has not been evaluated.

What is KALYDECO® (ivacaftor)?

KALYDECO is a prescription medicine used for the treatment of cystic fibrosis (CF) in people aged 1 month and older who have at least one mutation in the cystic fibrosis transmembrane conductance regulator (CFTR) gene that is responsive to KALYDECO.

Talk to your doctor to learn if you have an indicated CF gene mutation.

It is not known if KALYDECO is safe and effective in children under 1 month of age.

IMPORTANT SAFETY INFORMATION

People with CF pictured may or may not be taking KALYDECO.

Before taking KALYDECO, tell your doctor about all of your medical conditions, including if you:

- have liver or kidney problems
- are allergic to KALYDECO or any ingredients in KALYDECO. See the Patient Information for a list of ingredients

Please see Important Safety Information and full Prescribing Information, including Patient Information.

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Click below to get the information you're looking for.

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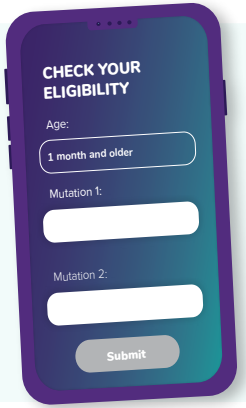

KALYDECO treats the underlying cause of cystic fibrosis (CF) in people age 1 month and older who have at least 1 CF gene mutation that is responsive to KALYDECO.

Visit KALYDECO.com/eligibility to see if you're eligible in 3 easy steps:

STEP 1: Enter your mutations.

STEP 2: Hit "Submit."

STEP 3: Discuss your results with your healthcare provider.

IMPORTANT SAFETY INFORMATION (Continued)

Before taking KALYDECO, tell your doctor about all of your medical conditions, including if you (continued):

- are pregnant or plan to become pregnant. It is not known if KALYDECO will harm your unborn baby. You and your doctor should decide if you will take KALYDECO while you are pregnant
- are breastfeeding or planning to breastfeed. It is not known if KALYDECO passes into your breast milk. You and your doctor should decide if you will take KALYDECO while you are breastfeeding

Tell your doctor about all the medicines you take, including prescription and over-the-counter medicines, vitamins, and herbal supplements.

KALYDECO may affect the way other medicines work, and other medicines may affect how KALYDECO works. Ask your doctor or pharmacist for a list of these medicines if you are not sure.

What is the history of KALYDECO® (ivacaftor)?

KALYDECO has been studied in multiple trials in specific age groups and with specific cystic fibrosis (CF) gene mutations.

Approved since
2012

KALYDECO was initially approved in 2012 for people age 6 years and older with a G551D mutation.

97
mutations

KALYDECO is indicated for 97 specific mutations based on studies with KALYDECO in clinical and/or laboratory settings.

Age **1+**
months

As of 2023, KALYDECO has been approved to treat people as young as 1 month who have at least one mutation in the CF gene that is responsive to KALYDECO.

IMPORTANT SAFETY INFORMATION (Continued)

Tell your doctor about all the medicines you take (continued)

Especially tell your doctor if you take:

- the antibiotics rifampin (RIFAMATE®, RIFATER®) or rifabutin (MYCOBUTIN®)
- seizure medicines such as phenobarbital, carbamazepine (TEGRETOL®, CARBATROL®, EQUETRO®), or phenytoin (DILANTIN®, PHENYTEK®)
- St. John's wort
- antifungal medicines such as ketoconazole, itraconazole (SPORANOX®), posaconazole (NOXAFIL®), voriconazole (VFEND®), or fluconazole (DIFLUCAN®)
- antibiotics such as telithromycin, clarithromycin (BIAXIN®), or erythromycin (ERY-TAB®)

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What is the underlying cause of cystic fibrosis (CF)?

CF is caused by CFTR protein defects. A mutation in the genes of a person with CF may make defective CFTR proteins that:

- Don't open correctly
- Don't get to the cell surface, where they are normally located

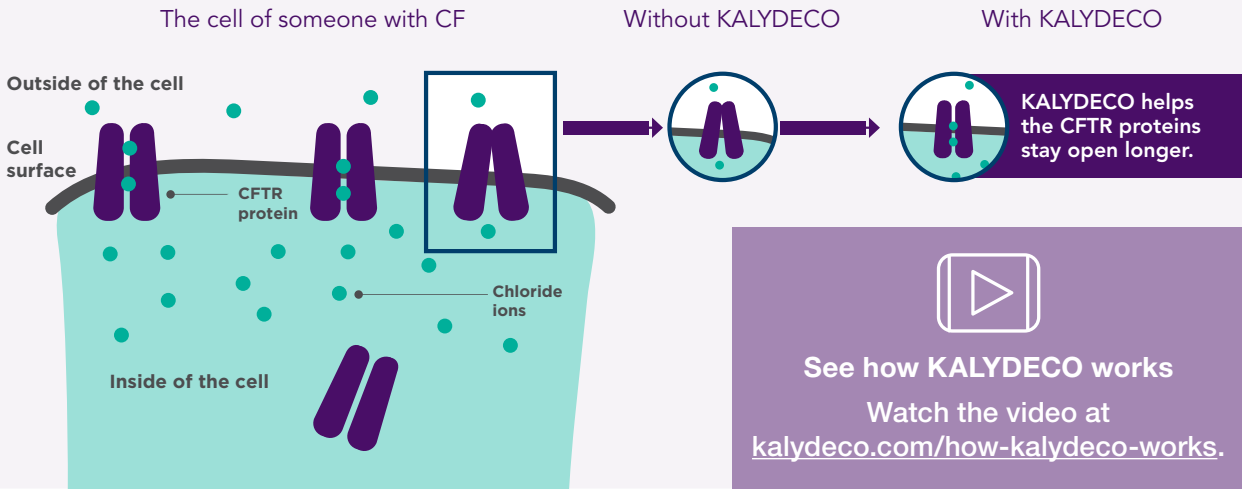
A person with CF may make CFTR proteins that have either or both of these defects.

Because of these defects, chloride ions cannot flow freely into or out of the cells as they should. This can lead to thick, sticky mucus in the lungs.

KALYDECO works on a certain defect of the CFTR protein at the cellular level.

KALYDECO allows more chloride ions to pass into and out of the cells, which may help keep a balance of salt and water in certain organs, such as the lungs. KALYDECO does not increase the number of CFTR proteins at the cell surface.

What is known about how KALYDECO works was learned from studies conducted in a lab. Keep in mind that results from laboratory studies do not always match how these medicines work in a person. If you have any questions about your KALYDECO treatment, please speak with your healthcare provider.



See how KALYDECO works

Watch the video at kalydeco.com/how-kalydeco-works.

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Before taking KALYDECO, tell your doctor about all of your medical conditions, including if you:

- have liver or kidney problems
- are allergic to KALYDECO or any ingredients in KALYDECO. See the Patient Information for a list of ingredients
- are pregnant or plan to become pregnant. It is not known if KALYDECO will harm your unborn baby. You and your doctor should decide if you will take KALYDECO while you are pregnant
- are breastfeeding or planning to breastfeed. It is not known if KALYDECO passes into your breast milk. You and your doctor should decide if you will take KALYDECO while you are breastfeeding

Tell your doctor about all the medicines you take, including prescription and over-the-counter medicines, vitamins, and herbal supplements.

KALYDECO may affect the way other medicines work, and other medicines may affect how KALYDECO works. Ask your doctor or pharmacist for a list of these medicines if you are not sure.

Especially tell your doctor if you take:

- the antibiotics rifampin (RIFAMATE®, RIFATER®) or rifabutin (MYCOBUTIN®)
- seizure medicines such as phenobarbital, carbamazepine (TEGRETOL®, CARBATROL®, EQUETRO®), or phenytoin (DILANTIN®, PHENYTEK®)
- St. John's wort
- antifungal medicines such as ketoconazole, itraconazole (SPORANOX®), posaconazole (NOXAFIL®), voriconazole (VFEND®), or fluconazole (DIFLUCAN®)
- antibiotics such as telithromycin, clarithromycin (BIAXIN®), or erythromycin (ERY-TAB®)

What should I avoid while taking KALYDECO?

- KALYDECO can cause dizziness in some people who take it. If you experience dizziness, do not drive or operate machines until symptoms improve
- Avoid food or drink containing grapefruit while you are taking KALYDECO

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What are the possible side effects of KALYDECO?

KALYDECO can cause serious side effects, including:

- **High liver enzymes in the blood,** which have happened in people receiving KALYDECO. Your doctor will do blood tests to check your liver:
  - before you start KALYDECO
  - every 3 months during your first year of taking KALYDECO
  - every year while you are taking KALYDECO

For people who have had high liver enzymes in the past, your doctor may do blood tests to check the liver more often.

Call your doctor right away if you have any of the following symptoms of liver problems:

- pain or discomfort in the upper right stomach (abdominal) area
- yellowing of your skin or the white part of your eyes
- loss of appetite
- nausea or vomiting
- dark, amber-colored urine

- **Serious allergic reactions** have happened to people who are treated with KALYDECO. Call your healthcare provider or go to the emergency room right away if you have symptoms of an allergic reaction. Symptoms of an allergic reaction may include:
  - rash or hives
  - tightness of the chest or throat or difficulty breathing
  - light-headedness or dizziness

- **Abnormality of the eye lens (cataract),** which has happened in some children and adolescents receiving KALYDECO. Your doctor should perform eye examinations before and during treatment with KALYDECO to look for cataracts.

The most common side effects of KALYDECO include:

- headache
- upper respiratory tract infection (common cold), including:
  - sore throat
  - nasal or sinus congestion
  - runny nose
- stomach (abdominal) pain
- diarrhea
- rash
- nausea
- dizziness

Tell your doctor if you have any side effect that bothers you or that does not go away. These are not all the possible side effects of KALYDECO. For more information, ask your doctor or pharmacist.

Use of KALYDECO in children aged 1 month to less than 6 months born from a pregnancy lasting (gestational age) less than 37 weeks has not been evaluated.

Call your doctor for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088.

Children with CF age 1 month to less than 4 months old

The safety and tolerability of KALYDECO® (ivacaftor) were evaluated in 43 children age 1 month to less than 2 years old with cystic fibrosis (CF). All the children in this study took KALYDECO.

Age 1 month to < 4 months

One part of the study evaluated 7 children **age 1 month to less than 4 months old**.

Mutations eligible to enroll in this part of the study were:

Any mutation responsive to KALYDECO.

See study results on the next page

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Study results

Safety

The safety of KALYDECO® (ivacaftor), observed in this study, was similar to what was observed in KALYDECO studies in people with CF age 2 years and older.

For safety information and side effects of KALYDECO, see pages 6-7.

Sweat chloride

For children age 1 month to less than 4 months old who took KALYDECO:

Sweat chloride decreased on average by -40.3 mmol/L at Week 24.

Sweat chloride is the amount of salt in a person's sweat. A decrease in sweat chloride levels does **not** mean there will be an improvement in lung function (FEV_1*).

*FEV_1=forced expiratory volume, or how much air a person can exhale in a forced breath in 1 second.

Study limitations

Because no one in this study took placebo, it is not known if changes in sweat chloride were due to KALYDECO.

KALYDECO was approved in children age 1 month to less than 4 months old based on the benefits shown in studies of KALYDECO in people age 6 years and older, as well as the safety assessment in this study. For more information on these studies, visit KALYDECO.com/study-results.

Talk to your healthcare provider to learn more about how KALYDECO was approved in this age group.

Children with CF age 4 months to less than 6 months old

The safety and tolerability of KALYDECO® (ivacaftor) were evaluated in 43 children age 1 month to less than 2 years old with cystic fibrosis (CF). All the children in this study took KALYDECO.

Age 4 months to < 6 months

One part of the study evaluated 6 children **age 4 months to less than 6 months old**.

Mutations eligible to enroll in this part of the study were:

G1244E, G1349D, G178R, G551D, G551S, R117H, S1251N, S1255P, S549N, S549R.

How KALYDECO was given

All children age 4 months to less than 6 months old received **25 mg of KALYDECO oral granules**.

In the clinical study, instruction was provided to give the dose of KALYDECO oral granules mixed into 1 teaspoon of an age-appropriate soft food or liquid every 12 hours along with a fat-containing food.

Learn more about the recommended dose of KALYDECO oral granules and how to give them to your loved one, starting on page 16.

See study results on the next page ▶

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Please see Important Safety Information and full Prescribing Information, including Patient Information.

Study results

Safety

The safety of KALYDECO® (ivacaftor), observed in this study, was similar to what was observed in KALYDECO studies in people with CF age 2 years and older.

For safety information and side effects of KALYDECO, see pages 6-7.

Sweat chloride

For children age 4 months to less than 6 months old who took KALYDECO:

Sweat chloride decreased on average by -50.0 mmol/L at Week 24.

Sweat chloride is the amount of salt in a person's sweat. A decrease in sweat chloride levels does **not** mean there will be an improvement in lung function (FEV_1*).

*FEV_1=forced expiratory volume, or how much air a person can exhale in a forced breath in 1 second.

Study limitations

Because no one in this study took placebo, it is not known if changes in sweat chloride were due to KALYDECO.

KALYDECO was approved in children age 4 months to less than 6 months old based on the benefits shown in studies of KALYDECO in people age 6 years and older, as well as the safety assessment in this study. For more information on these studies, visit KALYDECO.com/study-results.

Talk to your healthcare provider to learn more about how KALYDECO was approved in this age group.

Children with CF age 6 months to less than 1 year old

The safety and tolerability of KALYDECO® (ivacaftor) were evaluated in 43 children age 1 month to less than 2 years old with cystic fibrosis (CF). All the children in this study took KALYDECO.

Age 6 months to < 1 year

One part of the study evaluated 11 children **age 6 months to less than 1 year old**.

Mutations eligible to enroll in this part of the study were:

G1244E, G1349D, G178R, G551D, G551S, R117H, S1251N, S1255P, S549N, S549R.

How KALYDECO was given

All children in the study weighed **7 kg (~15 lb) to less than 14 kg (~31 lb)** and received **50 mg of KALYDECO oral granules**.

In the clinical study, instruction was provided to give the dose of KALYDECO oral granules mixed into 1 teaspoon of an age-appropriate soft food or liquid every 12 hours along with a fat-containing food.

Learn more about the recommended dose of KALYDECO oral granules and how to give them to your loved one, starting on page 16.

See study results on the next page ▶

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Study results

Safety

The safety of KALYDECO® (ivacaftor), observed in this study, was similar to what was observed in KALYDECO studies in people with CF age 2 and older.

For safety information and side effects of KALYDECO, see pages 6-7.

Sweat chloride

For children age 6 months to less than 1 year old who took KALYDECO:

Sweat chloride decreased on average by -58.6 mmol/L at Week 24.

Sweat chloride is the amount of salt in a person's sweat. A decrease in sweat chloride levels does **not** mean there will be an improvement in lung function (FEV_1*).

*FEV_1=forced expiratory volume, or how much air a person can exhale in a forced breath in 1 second.

Study limitations

Because no one in this study took placebo, it is not known if changes in sweat chloride were due to KALYDECO.

KALYDECO was approved in children age 6 months to less than 1 year old based on the benefits shown in studies of KALYDECO in people age 6 years and older, as well as the safety assessment in this study. For more information on these studies, visit KALYDECO.com/study-results.

Talk to your healthcare provider to learn more about how KALYDECO was approved in this age group.

Children with CF age 1 year to less than 2 years old

The safety and tolerability of KALYDECO® (ivacaftor) were evaluated in 43 children age 1 month to less than 2 years old with cystic fibrosis (CF). All the children in this study took KALYDECO.

Age 1 year to < 2 years

One part of the study evaluated 19 children **age 1 year to less than 2 years old**.

Mutations eligible to enroll in this part of the study were:

G1244E, G1349D, G178R, G551D, G551S, R117H, S1251N, S1255P, S549N, S549R.

How KALYDECO was given

Children who weighed **7 kg (~15 lb) to less than 14 kg (~31 lb)** received **50 mg of KALYDECO oral granules**.

Children who weighed **14 kg (~31 lb) or more** received **75 mg of KALYDECO oral granules**.

In the clinical study, instruction was provided to give the dose of KALYDECO oral granules mixed into 1 teaspoon of an age-appropriate soft food or liquid every 12 hours along with a fat-containing food.

Learn more about the recommended dose of KALYDECO oral granules and how to give them to your loved one, starting on page 16.

See study results on the next page ▶

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Study results

Safety

The safety of KALYDECO® (ivacaftor), observed in this study, was similar to what was observed in KALYDECO studies in people with CF age 2 and older.

For safety information and side effects of KALYDECO, see pages 6-7.

Sweat chloride

For children age 1 year to less than 2 years old who took KALYDECO:

Sweat chloride decreased on average by -73.5 mmol/L at Week 24.

Sweat chloride is the amount of salt in a person's sweat. A decrease in sweat chloride levels does **not** mean there will be an improvement in lung function (FEV_1*).

*FEV_1=forced expiratory volume, or how much air a person can exhale in a forced breath in 1 second.

Study limitations

Because no one in this study took placebo, it is not known if changes in sweat chloride were due to KALYDECO.

KALYDECO was approved in children age 1 year to less than 2 years old based on the benefits shown in studies of KALYDECO in people age 6 years and older, as well as the safety assessment in this study. For more information on these studies, visit KALYDECO.com/study-results.

Talk to your healthcare provider to learn more about how KALYDECO was approved in this age group.

What is the recommended dose for my loved one?

If your loved one is:	And weighs:	The recommended dose is:
1 month to less than 2 months old	**3 kg or more** *(~7 lb or more)*	**One 5.8-mg packet every 12 hours** Total daily dose: 11.6 mg
2 months to less than 4 months old	**3 kg or more** *(~7 lb or more)*	**One 13.4-mg packet every 12 hours** Total daily dose: 26.8 mg
4 months to less than 6 months old	**5 kg or more** *(~11 lb or more)*	**One 25-mg packet every 12 hours** Total daily dose: 50 mg
6 months to less than 6 years old	**5 kg to less than 7 kg** *(~11 lb to less than ~15 lb)*	**One 25-mg packet every 12 hours** Total daily dose: 50 mg
	7 kg to less than 14 kg *(~15 lb to less than ~31 lb)*	**One 50-mg packet every 12 hours** Total daily dose: 100 mg
	14 kg or more *(~31 lb or more)*	**One 75-mg packet every 12 hours** Total daily dose: 150 mg

Your healthcare provider may give you different instructions on how much KALYDECO® (ivacaftor) to give your loved one and when you should give it.

It is not known if KALYDECO is safe and effective in children under 1 month of age.

Use of KALYDECO in children aged 1 month to less than 6 months born from a pregnancy lasting (gestational age) less than 37 weeks has not been evaluated.

3 steps for giving your loved one KALYDECO oral granules

STEP 1: Preparation

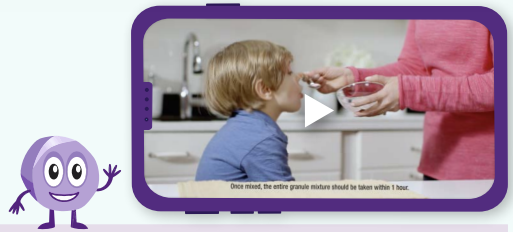
- Hold 1 packet of KALYDECO oral granules with the cut line on top
- Shake the packet gently to settle the KALYDECO granules
- Tear or cut the packet open along the cut line
- Carefully pour all of the KALYDECO granules into 1 teaspoon (5 mL) of age-appropriate soft food or liquid and mix

Not actual size. Your loved one's KALYDECO carton and packets may look different.

Here are some examples of soft foods and liquids you can mix KALYDECO granules into. Remember, every child is different, so be sure to talk to your healthcare provider about what the best options are for your loved one.

- Breast milk
- Infant formula
- Puréed fruits or vegetables
- Applesauce
- Water
- Milk
- Yogurt
- Juice

- Food or liquid should be at or below room temperature
- Even if the food or liquid you mix the granules into contains fat, you still need to give your loved one fat-containing food. See steps 2 and 3 on the next page



Join Grant the Granule and a family as they learn each step for preparing KALYDECO. Visit KALYDECO.com/granules.

Continued ▶

STEP 2: Administration

- Give KALYDECO to your loved one within one hour of mixing
- Make sure the entire medicine mixture is taken

STEP 3: Fat-containing food before or after the dose

Always give your loved one KALYDECO with a fat-containing food

- Give your loved one fat-containing food just before or just after the dose of KALYDECO granules. This helps the body absorb KALYDECO better

Here are suggestions for fat-containing foods to give your loved one. Please discuss with your healthcare provider what may be age-appropriate.

- Breast milk
- Whole milk
- Eggs
- Butter
- Cheese pizza
- Infant formula
- Whole-milk yogurt
- Whole-milk cheese
- Peanut butter

Avoid foods and drinks that contain grapefruit—these may affect the amount of KALYDECO in the body.

Everyday CF

Get delicious recipes and food ideas on **Everyday-CF.com**.

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What to do if a dose of KALYDECO is missed

If a dose of KALYDECO is missed and it is **within 6 hours** of when it is usually given, give that dose of KALYDECO as prescribed with fat-containing food as soon as possible.

If a dose of KALYDECO is missed and it is **more than 6 hours** after the time the dose is usually given, **skip that dose only** and give the next dose with fat-containing food when it is usually given. Do **not** give 2 doses at the same time to make up for a missed dose.

Remember:

- Giving KALYDECO every 12 hours is not the same as giving it twice a day
- Give KALYDECO, plus all other cystic fibrosis (CF) therapies, exactly as your loved one's healthcare provider tells you
- Tell your loved one's healthcare provider before starting, changing, or stopping any medicines your loved one takes, including prescription and non-prescription medicines, vitamins, and herbal supplements

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Q: Do breast milk and prepared infant formula qualify as fat-containing foods that I can give my loved one with KALYDECO?

Yes, breast milk and prepared infant formula qualify as fat-containing foods.

Q: If I mix the KALYDECO oral granules with a fat-containing food, do I still need to give my loved one fat-containing food afterwards?

Yes, your loved one should still eat fat-containing food just before or just after taking the entire mixture.

Q: Does the temperature of the food that I mix with KALYDECO oral granules matter? Can the granules be mixed in foods that are hot or cold?

The granules should be mixed with soft food or liquid at room temperature or below.

Q: What happens if my loved one gains weight and becomes eligible for a higher dose?

The dose of KALYDECO is based on your loved one's age and weight. As your loved one gets older, your healthcare provider may adjust their dose depending on their weight.

Q: Can my loved one swallow the granules without mixing them in soft food or liquid?

The entire contents of each packet should be mixed with 1 teaspoon (5 mL) of age-appropriate soft food or liquid. The mixture should be taken within 1 hour of being mixed. Make sure all medicine is taken.

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Q: My loved one spit up their dose of KALYDECO oral granules. What should I do?

You should contact your loved one's healthcare provider to discuss this. Your pharmacist may also be a good resource.

Q: If my loved one is napping when it is time for KALYDECO oral granules, should I wait until they're awake to administer them?

You should give your loved one KALYDECO oral granules every 12 hours or as prescribed by your loved one's healthcare provider.

Q: Do the granules have a taste?

The granule formulation is sweetened but unflavored.

Q: How should KALYDECO oral granules be stored?

KALYDECO should be stored at 68°F to 77°F (20°C to 25°C).
Do not use KALYDECO after the expiration date on the package.
Keep KALYDECO and all medicines out of the reach of children.

Q: Are the oral granules packets child proof?

The packets are child-resistant.

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VERTEX GPS™
GUIDANCE & PATIENT SUPPORT

We're here to help you get there

Wherever life with cystic fibrosis (CF) takes you, Vertex GPS™: Guidance & Patient Support is here to help. We offer personalized, one-on-one support to help you start and stay on track with treatment. Once you're enrolled, you'll be assigned a dedicated Support Specialist who will be with you every step of the way.

Here are just some of the ways your Support Specialist can help:

Get you started on treatment by verifying your coverage and out-of-pocket costs with your **insurance company**. They'll also connect with your **healthcare provider** to discuss any requirements or questions your insurance company may have while determining coverage.

Help you explore financial assistance options, regardless of your insurance coverage. And if you have commercial insurance, the Vertex GPS Co-pay Assistance Program may be able to lower your co-pay to as little as $0 per fill.*

*Eligibility restrictions and limitations apply. Annual assistance is limited to a maximum of $20,000.

Keep you on track with your treatment by coordinating shipments with your **specialty pharmacy** and reminding you when it's time to refill your Vertex medicine. And if your daily routine changes, they can help you pre-plan refills, ship your medicine to a new address, and share tips to help you stay motivated.

Meet your everyday needs with information on nutrition and tips for staying physically active and maintaining a healthy mindset. And if you're caring for someone with CF, they'll send educational resources to help you teach your loved one about the importance of their daily treatment routine.

Plan for what's ahead as you approach big life changes. They can help you prepare for your next chapter and give you tips on staying on track with treatment. They can also share advice from others living with CF.

Not enrolled in Vertex GPS?

If you have been prescribed a Vertex medicine, ask your healthcare provider to complete an enrollment form for you.

Already enrolled?

If you are currently enrolled in GPS, you can call or text your Support Specialist at **1-877-752-5933 (press 2 when calling)**, Monday through Friday, from 8:30 AM to 7 PM ET.

Discover more about GPS and the support resources available at VertexGPS.com.

VERTEX